MSC delivery of an artificial transcription factor to the brain as a treatment for Angelman Syndrome

Grant Award Details

MSC delivery of an artificial transcription factor to the brain as a treatment for Angelman Syndrome

Grant Type: Quest - Discovery Stage Research Projects

Grant Number: DISC2-09032

Project Objective: MSC-delivered artificial transcription factor to the brain as a treatment for Angelman Syndrome

Investigator:

Name:	David Segal
Institution:	University of California, Davis
Type:	PI

Disease Focus: Autism, Neurological Disorders

Human Stem Cell Use: Adult Stem Cell

Award Value: $1,087,572

Status: Active

Grant Application Details

Application Title: MSC delivery of an artificial transcription factor to the brain as a treatment for Angelman Syndrome

Public Abstract:

Research Objective

Mesenchymal stem cells will be used to deliver an artificial transcription factor to neurons in the brain to treat a genetic disease.

Impact

It could lead directly to a treatment for Angelman Syndrome, but the approach could be used to alter gene expression in almost any brain disorder. It could overcome the brain delivery bottleneck.

Major Proposed Activities

- Prepare the MSC delivery system (month 1 – month 6)
- Rescue and analysis of on-target molecular phenotypes in "YFP-mice" (month 6 – month12)
- Rescue and analysis of the behavioral phenotypes in "AS-mice" (month 12 – month 24)
- Analysis of the off-target molecular phenotypes in "YFP-mice" (month 18 – month 24)

Statement of Benefit to California:

Brain disorders are responsible for more years lost to disability than any other medical condition. For example, autism spectrum disorder (ASD) in the US is estimated to affect 1 in 68 children. The need for effective treatments can not be understated. Molecular therapeutics pioneered to understand and treat rare single-gene disorders such as Angelman Syndrome will provide the tools and methods that will ultimately be used to address the more common complex brain disorders.
